

Controversial muscular dystrophy therapy goes to court

This July, a Memphis-based clinic called the Cell Therapy Research Foundation (CTRF) filed a legal "complaint" against some of the most respected researchers in the field of muscular dystrophy (MD). The CTRF, which specializes in treating muscular dystrophies, is seeking \$1 million compensation and a \$10 million award for punitive damages for libelous and slanderous remarks about its clinical practices. CTRF says the money will be used to create a research fund for two of the most severe forms of the disease — Becker and Duchenne MD.

The defendants in the action are Terrence Partridge of London's Royal Postgraduate Medical School, a senior researcher and 25-year veteran in the field of muscular disease, and Eric Hoffman of the University of Pittsburgh, a member of the 1987 team that discovered dystrophin (the muscle protein known to be missing in this disease group). Brazilian researcher Mayana Zatz and Koren Stark of Redmond, Washington — the mother of a Duchenne muscular dystrophy boy — are also named in the proceedings.

CTRF chairman Peter Law, who has worked in the field for as long as Partridge, cites a Pittsburgh University website as one place where notices advising parents of stricken boys not to take their sons to Law's clinic for myoblast transfer therapy (MTT) were posted. The therapy is purported to increase muscle function in MD patients and costs around \$150,000. The offending article has since been removed.

The filing represents the climax of a seven-year dispute between Law and other MD researchers which spilled into print this summer when a series of open letters were exchanged in the journal, *Cell Transplantation*. Partridge and his colleagues asked for an independent analysis to be performed on biopsy material from selected patients to answer some of the issues surrounding the MTT procedure. Although major national charities concerned with neuromuscular diseases offered to under-

write the cost of this evaluation, no tissue has yet been released from the CTRF.

Law's therapeutic technique involves the injection of 50 billion donor myoblasts into 82 muscle groups. In theory, the myoblasts fuse with host cells and express functional dystrophin protein. Law, who has treated more than 120 pa-

idated." Charash adds that, having known Law before he started human experimentation, he has seen "not a bit of evidence" that the therapy has benefited anyone.

Furthermore, scientists in the field offer many reasons why the technique cannot possibly work. One is that myoblasts migrate poorly from the site of injection, making it doubtful that they would reach sufficient muscle cells, including heart and diaphragm muscles, for the therapy to be effective. Others point to the short-lived dystrophin expression of injected myoblasts and say that Law does not examine tissue for dystrophin production after treatment, and so he has no idea what the efficiency of delivery is.

Law refers his critics to data published in peer-reviewed journals and presented at major conferences for proof that his approach is a valid one. He says that the protocol has endured the scrutiny of the most stringent review board in the country — the FDA — and this should be a sufficient guarantee. Furthermore, he is hoping to receive FDA approval for a 50-patient Phase III multicenter study to begin early next year. To support this, he recently asked the MDA for \$4.2 million in funding: they replied that they *will* give the money on the condition that a single patient is treated and evaluated under their observation.

One of the defendants in the case told *Nature Medicine* that while they regret the expense and time taken up by a judicial process, the real price they pay is the loss of freedom for scientific debate on the issue. They are hopeful that a court case will at least force Law and his technique out into the open for examination.

KAREN BIRMINGHAM

Muscular Dystrophy Patient Walks After Years in a Wheelchair Following 50 Billion Cell Transplant

In the most dramatic breakthrough yet in the fight against muscular dystrophy, a 29-year-old North Carolina man who received an experimental cell transplant took his first steps after being confined to a wheelchair for years. Recently, David has been able to walk unassisted on crutches. He continues to improve, much to the delight of family, friends and well-wishers.

David Plemons suffers from Becker muscular dystrophy. He received US Food and Drug Administration supervised myoblast transfer therapy (MTT) on January 28, 1997, as part of Memphis-based Cell Therapy Research Foundation's (CTRF) Phase I clinical trials. MTT involves transplanting 50 billion muscle cells called "myoblasts" into 82 muscle groups in a single, 45 minute procedure.

Just over two months after the transplant, David took his first assisted steps.

"This was the most important day in my life" says David. "I thought I would have to spend the rest of my life in a wheelchair, but now I'm able to walk with minimal assistance of his parents and girlfriend. I've got a new shot at life."

"This is the culmination of 28 years' work," said Dr. Peter Law, CTRF's Founder and Chairman. "We are overjoyed for David. Our goal is to develop myoblast transfer therapy to treat people throughout the world that are stricken with genetic diseases," he said. The CTRF team comprises 30 professionals and staff.

Muscular dystrophy leads to the progressive degeneration of muscles. In the most severe forms, it leads to early death. In Becker muscular dystrophy it confines the patient to a wheelchair leading to death at 40.

MTT involves transplanting immature muscle cells, which carry a full complement of normal genes from a healthy donor into the muscles of an individual with muscular dystrophy. The transplanted cells repair and replace degenerating cells in the dystrophic individual. The new muscles then produce the missing protein known as dystrophin, allowing the muscles to gain strength. In theory, enough muscle cells could be transplanted into a patient to reverse

tients from 17 countries, claims the technique is so successful that it has enabled a previously wheelchair-bound patient with Becker's MD to walk again. This success story is depicted on the CTRF website.

However, criticism of the method is widespread. Leon Charash, chairman of the national medical advisory committee to the Muscular Dystrophy Association (MDA), says that Law is making "repeated claims of dramatic improvement that have never been val-

Medical students accused of cheating

An anonymous letter and suspicious testing patterns led the National Board of Medical Examiners (NBME) in the US to postpone notification of results from part one of the US Medical Licensing Exam to 20,000 students. Announcements were delayed by eleven days in August while allegations of cheating were investigated. The exam is administered to students about to enter their third year of medical school and the scores later affect their placement

in residency programs. It is thought that some students obtained advance copies of the exam. The investigation is ongoing, with a focus on non-US medical schools — rumor suggests that students at an Asian medical school were selling the test. Those found cheating could be banned from taking the exam again and may face criminal charges, according to NBME president Thompson Bowles.

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